

DEPARTMENT OF HEALTH AND HUMAN SERVICES
NATIONAL INSTITUTES OF HEALTH

Budget Request for FY 2010

Witness appearing before the
Senate Subcommittee on Labor-HHS-Education Appropriations

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Mr. Chairman and Members of the Committee:

Thank you for the opportunity to offer testimony on behalf of the National Cancer Institute (NCI) and the National Cancer Program.

I am pleased to present the President's Fiscal Year 2010 Budget request for the National Cancer Institute (NCI) of the National Institutes of Health (NIH). The FY 2010 budget includes \$5,150,170,000, which is \$181,197,000 more than the FY 2009 appropriation of \$4,968,973,000.

DOUBLING CANCER RESEARCH

The FY 2010 Budget reflects the President's prioritization of biomedical research supported by NIH. The Budget is the first year of an eight-year strategy to double the NIH-wide cancer research budget and includes over \$6 billion for this purpose. The Budget balances the President's commitment to cancer research with that of research in other areas.

NIH's FY 2010 Budget will build upon the unprecedented \$10 billion provided in the American Recovery and Reinvestment Act of 2009, which will support new NIH research on a wide array of diseases, condition, and disorders in 2009 and 2010.

Because cancer research involves the dissection and understanding of perhaps the most basic functions of human cell growth and differentiation, cancer research will always produce many serendipitous discoveries. Such discoveries involving the most basic properties of human cells have historically contributed to our understanding of the basic biology underlying almost all diseases.

In addition, cancer research also involves technology development that will benefit research in a number of disease areas. For example, cancer research includes a major effort to understand the complete genetic alterations that result in abnormal cell growth.

This effort in whole genome sequencing is a major driver in the development of sequencing technology that we believe will lead to our ability in the next 2-3 years to perform whole genome sequencing in a matter of hours for less than \$1,000.

Numerous other Institutes and Centers contribute their expertise to fundamental research on biological processes, technologies and tools, and work collaboratively with NCI to fund important research in cancer. For example, much of what has been learned at NCI in controlling tobacco usage is now being applied to study and address the growing health burden of obesity. NIH will work to ensure that cancer research resources are allocated responsibly, effectively, in accordance with peer review principles, and on the basis of sound science and cancer relevance.

MOVING PAST A LEGACY OF FEAR

One of the great American voices on behalf of biomedical research was Mary Lasker. A well-known figure in Washington politics and government, Mrs. Lasker was a driving force behind the creation of several Institutes of the National Institutes of Health and a key player in the formulation and passage of the National Cancer Act of 1971. Among her towering accomplishments, however, one stands out, perhaps because of its simplicity. In the years after World War II, cancer, she once remarked, remained “a word you simply could not say out loud.” Mary Lasker changed that. She persuaded David Sarnoff, the powerful head of the Radio Corporation of America – RCA – to allow the utterance on the airwaves of that single, chilling word.

Today, we feel no compulsion to avoid speaking its name; yet few would argue that we fear cancer less in 2009 than we did 50 or 100 years ago. Cancer will befall approximately one of two American men and one of three American women. Its diagnosis engenders thoughts of mortality, of debilitating treatments, of diminished quality of life, of lingering burdens on loved ones, of personal financial peril.

This major health problem is fueled by an aging, more heterogeneous population. A study published in April 2009 by the University of Texas M.D. Anderson Cancer Center estimated that the number of new cancer cases in the United States each year will increase by 45 percent over the next two decades, to 2.3 million per year by 2030.

It is thus quite understandable when the public and those responsible for health care ask if we are investing enough to advance the science needed to avert such predictions. Since 1971, the federal government, private foundations, and companies have spent approximately \$200 billion on cancer research. This investment has led to our understanding of many of cancer's numerous complexities; has resulted in a steady decline in the annual overall cancer mortality – and has increased the number of cancer survivors to more than 12 million Americans. NCI's budget request and its research projects are consistent with the President's multi-year commitment for Cancer and Autism. Aggressive programs in screening and prevention have greatly reduced the incidence of a number of cancers. For example, NCI led efforts to eliminate the use of tobacco has resulted in a 1.9 percent decrease per year from 1992 to 2003 in male lung cancer incidence rates. This has accelerated to a 3.3 percent decline per year over the period from 2003 to 2006. Despite these advances, it is evident that a greater investment than ever is needed to continue the dissection of the fundamental biology underlying the initiation of abnormal cell growth and its progression to invasive and metastatic disease.

THE POWER OF THE GENOME

Cancer is an extremely complex disease of altered genes. These changes within the cells of our body take many forms – and are both inherited and acquired, as we live out our lives. Since the completion of the Human Genome Project in 2003, the knowledge of the genetic alterations associated with cancer has grown exponentially. Vastly improved technologies are making it possible to study the genomes of thousands of individuals, in the search for common abnormalities that point to risk of cancer. Likewise, one of NCI's signature projects, The Cancer Genome Atlas (TCGA), is

studying the genetic changes associated with the development of several cancer types, including lung, ovarian, and brain cancers. The success of this pilot program is leading NCI to expand TCGA's scope to the sequencing of 20 to 25 tumor types. Sequencing these tumors in more than 200 patients per tumor type, coupled with whole genome scans of large population cohorts, is uncovering important information about cancer risk and patient-specific profiles unique to disease. Within just five years, some have suggested, whole genome deep sequencing will be part of virtually every laboratory cancer experiment, and within a decade, such deep genomic sequencing will be commonplace for patients.

At this moment, the results of this deep probing of the genetic basis of cancer remain, in most cases, fascinatingly powerful information. How we turn that information – sometimes referred to as code – into new methods of prevention, early detection, and treatment of cancer will require a major infusion of new resources. We must convert this coded information, which is stored in large data sets, into a clear interpretation and understanding of the functional biological alterations these genetic changes impart. NCI is working to fill this large gap in our knowledge, through a well-considered, coordinated blueprint appropriate for a new era of medicine. It begins with new discoveries at the level of the gene and ends at the patient's bedside.

NCI is preparing to bring together a network of investigators, whose work will begin after genomic sequencing is completed, taking information generated by TCGA and allied projects and turning that data into new knowledge of biologic function. The goal will be to identify potential new therapeutic targets in molecular pathways and physical processes that are, today, considered “undruggable.” This network will be virtual: a consortium of researchers primarily at research universities who will be offered the chance to participate in collaborative projects, often partnering between institutions. These projects will be prioritized on the basis of potential patient impact and technical feasibility – assigned to investigator sites on a competitive basis, each with a project manager.

The targets that will come forward from this functional biology consortium will be somewhat akin to a key piece of a jigsaw puzzle. It will be necessary to find the adjoining pieces – the new drugs, biologics, and other therapeutics – that connect. When potential new targets emerge, NCI will then employ its state-of-the art, high-throughput capacity to screen thousands of previously identified compounds, both natural and synthetic, to identify the exact piece to complete the puzzle.

In many cases, new therapies will require refinement, for example, to make them water soluble, or to create mass-producible versions of a natural product. Another virtual network, the Chemical Biology Consortium, will provide the necessary chemistry and chemists to optimize further development of these new anti-cancer agents. NCI will then be able to have those new agents produced in small batches for refinement and testing – using best manufacturing principles – and move them into pre-clinical testing, including toxicology screening.

Early phase clinical trials will follow. NCI has conducted the first of a new kind of trial called Phase 0, which uses a small number of carefully selected patients who, after receiving small doses of new drugs, are studied, in real time, at the molecular level, to see if the new medication is reaching and affecting its target. Phase 0 trials will allow for significantly earlier decisions on whether to move forward with Phase 1 trials.

It is not only Phase 0 trials that will require well characterized patients. As genomic characterization of the populace comes closer to becoming standard medical practice, NCI is taking steps on the leading edge of that transition, creating the first of a national network of patient characterization centers that will centrally conduct genomic and genetic characterization. Always employing the latest technologies and standardized protocols, these facilities will serve wide geographic areas, bringing together genomics and genetics, proteins and proteomics, all in the interest of matching a genetically characterized patient and his or her characterized tumor to appropriate and optimal therapeutic solutions.

The NIH Clinical Center, NCI's Specialized Programs of Research Excellence (SPORes), the NCI Community Cancer Centers Program; Cooperative Groups, the Community Clinical Oncology Program, and the NCI-designated Cancer Centers network will all be key players in establishing a highly-characterized national cohort of patients who can be easily matched with potential new agents.

DEVELOPING ELECTRONIC HEALTH RECORDS

Creating an integrated, 21st century translational science program will require data integration and a national commitment for the cancer electronic health record. NCI's cancer Biomedical Informatics Grid, better known as caBIG™, and its companion BIG Health Consortium, are leaders in this federal effort, working to develop a unified biomedical information infrastructure, along with data standards and protocols for electronic medical records that are consistent with the Federal government's national health IT efforts . Through caBIG, NCI is helping both large facilities from the NCI-designated Cancer Centers network and local facilities in the NCI Community Cancer Centers Program develop electronic records.

In addition, accomplishing the scale-up of TCGA and the genetic characterization of our patients – with data integration through caBIG – will require biospecimens collected using standardized protocols, tissue characterization, cataloging, and analysis, all coordinated by NCI's caHUB initiative.

A WIDE-RANGING EFFORT

This plan will require the contributions of biologists, chemists, informaticians, and clinical scientists devoted to a clear path from discovery to patient. This is not only the nature of translation; it will be a model for the study of many diseases and, ultimately, a model of 21st century healthcare. This platform is a vision for a new way of thinking. But it is not an unrealistic concept. It is an action plan: a roadmap for what we have begun to assemble this year, making the optimal use of every new resource.

In 2008, NCI began a series of meetings with theoretical physicists and mathematicians, designed to bring unique perspectives to the problem of cancer. The result is a new network of physical sciences-oncology centers, soon to launch, which will study physical forces – heat, stress, and cellular evolution, just to name a few – in cancer. This network is an exciting frontier in cancer research, which we fervently believe will be further proof that scientific collaboration pays great dividends.

NCI's goal is to make cancer a chronic condition one can live with, and not die from. We will continue to find better ways to prevent cancer's development and for the earliest detection, when a tumor is limited to a very small number of cells. We will continue to develop new therapies with fewer side-effects and greater quality of life. We will continue to study environmental causes of cancer. We will continue efforts to better understand the behaviors that increase cancer risk, and we will continue to follow those who have survived cancer, to understand the reasons why they are so often at risk for subsequent malignancies. These efforts will require coordinated programs and the continued work of a remarkable national cadre of individual laboratory investigators.

NCI is committed to paying dividends on behalf of every American. We no longer fear speaking the word cancer. Yet, our work is far from finished, and NCI remains committed to making every effort to advance a vastly different medical future.

Thank you for the opportunity to provide you this testimony. I look forward to the opportunity to take your questions.

John E. Niederhuber, M.D.,
Director, National Cancer Institute

John E. Niederhuber, M.D., became Director of the National Cancer Institute (NCI), one of the National Institutes of Health, in September 2006. Throughout his distinguished career in academic medicine, Dr. Niederhuber has maintained ties to both NCI and the NIH. In addition to his work as a cancer surgeon, professor, laboratory investigator, department chair, medical school senior associate dean, associate dean for research, and university cancer center director, Dr. Niederhuber has also served as the Chair of the National Cancer Advisory Board, as an external NCI advisor and grant reviewer, and as a laboratory investigator supported by NCI and the NIH.

He joined NCI in a full-time capacity in September 2005, as Deputy Director for Translational and Clinical Sciences, and within a few weeks was asked to serve as Chief Operating Officer. He officially became NCI's Acting Director in June 2006. Since assuming the directorship of the NCI, Dr. Niederhuber has shaped the Nation's investment in cancer, to address areas that are likely to pay the largest health dividends. Genomic studies of cancer and work in cancer immunotherapy, along with investigations into the tumor microenvironment, cancer initiating cells, and subcellular imaging have benefited under his direction.

Dr. Niederhuber is recognized by his peers as a visionary leader in oncology. As a leader of the National Cancer Program, he daily puts into practice his expertise as both a cancer physician and a basic research scientist. His colleagues have acknowledged his leadership and accomplishments by electing him vice president and president of the Society for Surgical Oncology and president of the Association of American Cancer Institutes. He has served as a member of C-Change (a community of executives from government, business, and the non-profit community dedicated to conquering cancer) and as a member of the CEO Roundtable on Cancer. Recently, Dr. Niederhuber was elected to the Institute of Medicine of the National Academy of Sciences, in recognition of his outstanding scientific accomplishments and commitment to service in health sciences.

In addition to his leadership of the NCI, Dr. Niederhuber heads his own Laboratory of Tumor and Stem Cell Biology in NCI's Center for Cancer Research and also holds a clinical appointment to the NIH Clinical Center Medical Staff. His lab is studying tissue stem cells as the cell-of-origin for cancer, as well as the complex relationship between tumor cells and their microenvironment.

As a surgeon, Dr. Niederhuber's clinical focus has been on gastrointestinal cancer, hepatobiliary (liver, bile duct, and gallbladder) cancer, pancreatic cancer and breast cancer. Recognized for his pioneering work in hepatic artery infusion chemotherapy, he was the first to demonstrate the feasibility of totally implantable vascular access devices which dramatically changed the administration of systemic chemotherapy.

Prior to coming to NCI, Dr. Niederhuber was Director of the University of Wisconsin Comprehensive Cancer Center and a professor of surgery and oncology (member of the McArdle Laboratory) at the University of Wisconsin School of Medicine. Earlier in his career, he chaired the Department of Surgery at Stanford University and held professorships at the Johns Hopkins University School of Medicine and at the University of Michigan.

A native of Steubenville, Ohio, Dr. Niederhuber is a graduate of Bethany College in West Virginia (receiving an honorary doctorate in 2007) and the Ohio State University School of Medicine.

Department of Health and Human Services
Office of Budget
Richard J. Turman

Mr. Turman is the Deputy Assistant Secretary for Budget, HHS. He joined federal service as a Presidential Management Intern in 1987 at the Office of Management and Budget, where he worked as a Budget Examiner and later as a Branch Chief. He has worked as a Legislative Assistant in the Senate, as the Director of Federal Relations for an association of research universities, and as the Associate Director for Budget of the National Institutes of Health. He received a Bachelor's Degree from the University of California, Santa Cruz, and a Masters in Public Policy from the University of California, Berkeley